

Efficacy of Nirogacestat in Patients With Desmoid Tumors and Presence or Absence of Poor Prognostic Factors: Post Hoc Analyses of the Phase 3 DeFi Trial

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SUPPLEMENTAL MATERIAL

Figure S1. PRO of physical functioning, role functioning, and overall quality of life

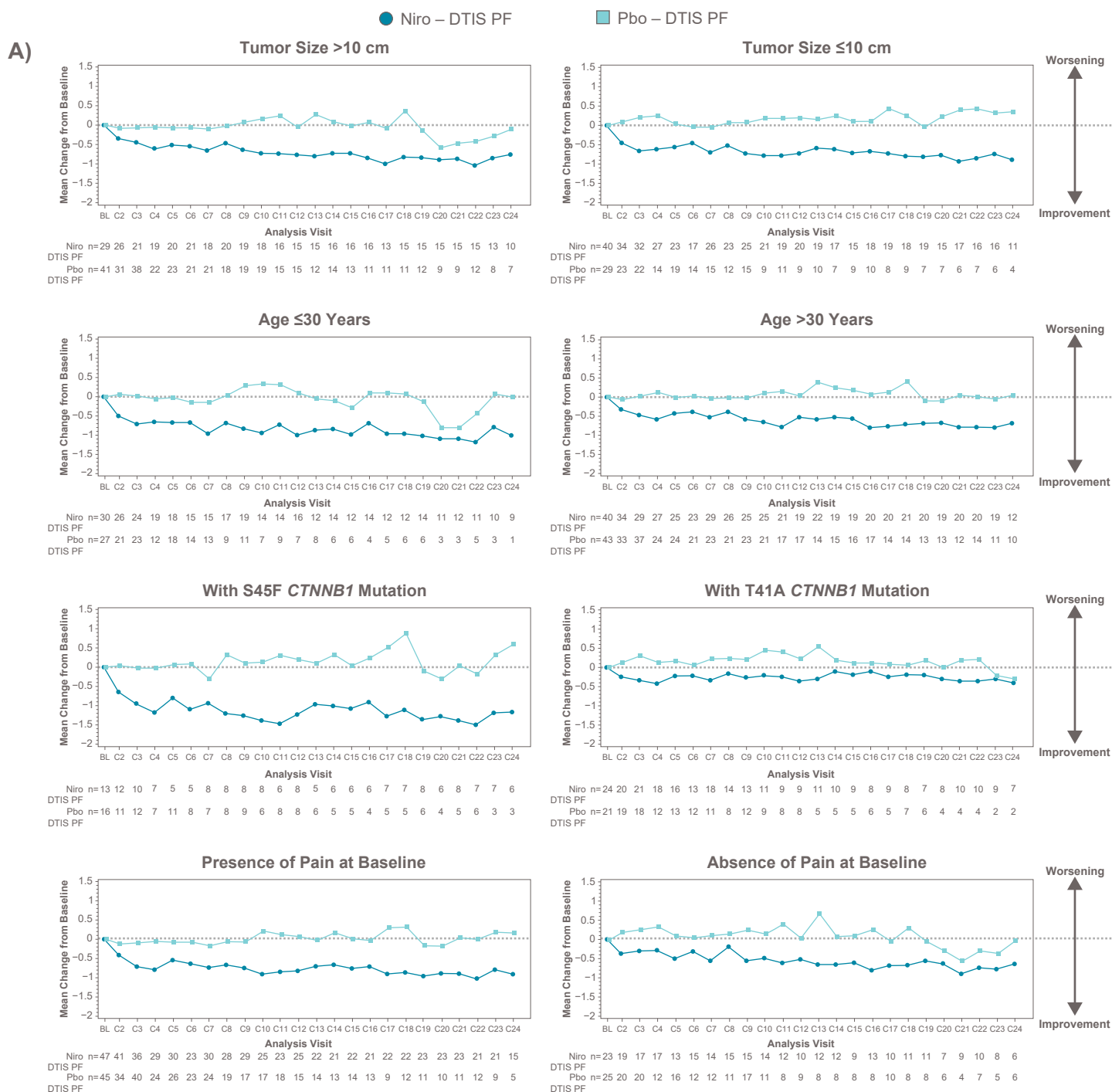
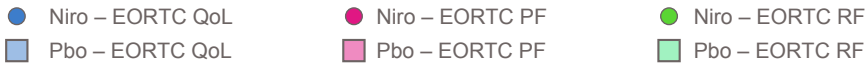
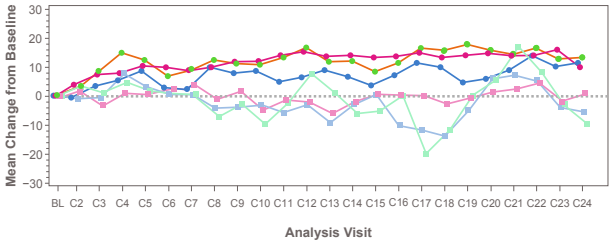


Figure S1. PRO of physical functioning, role functioning, and overall quality of life (cont.)



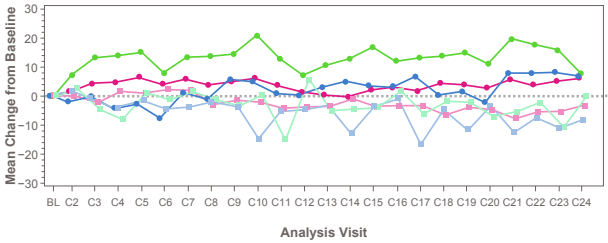
B)

Tumor Size >10 cm



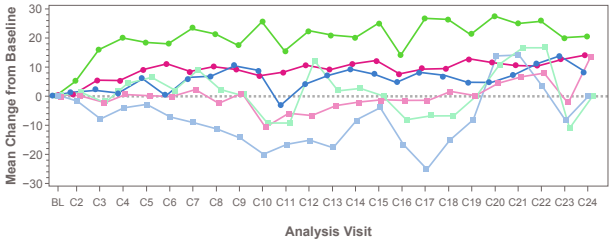
Niro EORTC QoL	n=29	26	21	19	20	21	18	20	19	18	16	14	14	16	16	16	13	15	14	15	15	13	10
Niro EORTC PF	n=29	26	21	19	20	21	18	20	19	18	16	14	14	16	16	16	13	15	14	15	15	13	10
Niro EORTC RF	n=29	26	21	19	20	21	18	20	19	18	16	14	14	16	16	16	13	15	14	15	15	13	10
Pbo EORTC QoL	n=40	30	37	21	22	21	17	18	18	15	14	11	13	13	9	10	10	9	8	7	11	7	6
Pbo EORTC PF	n=41	31	37	22	23	21	17	19	19	15	12	14	13	10	11	10	10	9	8	12	7	7	7
Pbo EORTC RF	n=41	31	37	22	23	21	17	19	19	15	12	14	13	10	11	10	10	9	8	12	7	7	7

Tumor Size ≤10 cm



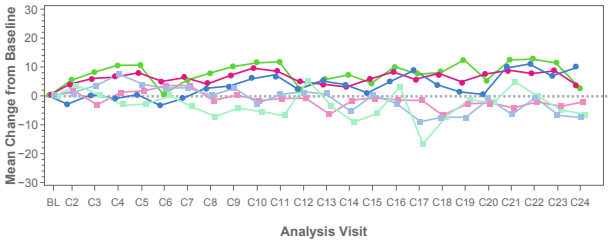
Niro EORTC QoL	n=40	34	32	27	23	17	25	22	25	20	17	19	19	17	15	18	18	18	19	15	17	16	11
Niro EORTC PF	n=40	34	32	27	23	17	25	23	25	20	18	19	19	17	15	18	19	18	19	15	17	16	11
Niro EORTC RF	n=40	34	32	27	23	17	25	23	25	20	18	19	19	17	15	18	19	18	19	15	17	16	11
Pbo EORTC QoL	n=29	21	22	14	18	14	15	12	14	9	11	9	10	7	9	10	7	9	7	7	6	7	4
Pbo EORTC PF	n=29	21	22	14	18	14	15	12	15	9	11	9	10	7	9	10	8	9	7	7	6	7	4
Pbo EORTC RF	n=29	21	22	14	18	14	15	12	15	9	11	9	10	7	9	10	8	9	7	7	6	7	4

Age ≤30 Years



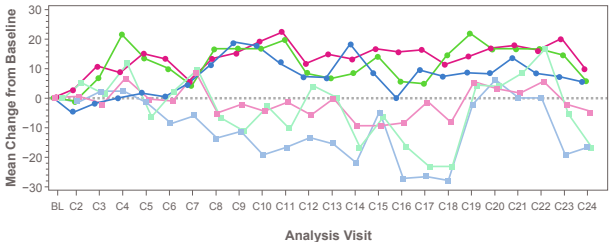
Niro EORTC QoL	n=30	26	24	19	17	15	16	19	13	13	15	12	14	12	14	11	12	11	10	9			
Niro EORTC PF	n=30	26	24	19	18	15	17	19	13	13	15	12	14	12	14	11	12	11	10	9			
Niro EORTC RF	n=30	26	24	19	18	15	17	19	13	13	15	12	14	12	14	11	12	11	10	9			
Pbo EORTC QoL	n=27	19	23	12	18	14	13	8	11	7	9	7	8	6	4	5	5	3	3	5	3	1	
Pbo EORTC PF	n=27	19	23	12	18	14	13	8	11	7	9	7	8	6	4	5	5	3	3	5	3	1	
Pbo EORTC RF	n=27	19	23	12	18	14	13	8	11	7	9	7	8	6	4	5	5	3	3	5	3	1	

Age >30 Years



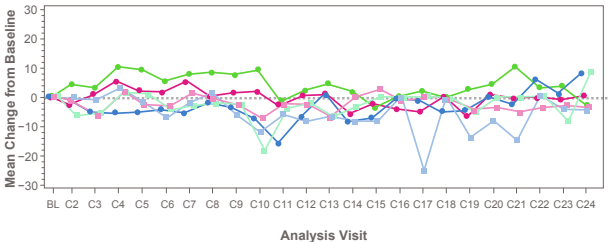
Niro EORTC QoL	n=40	34	29	26	25	23	28	26	25	25	20	18	21	19	19	20	20	19	19	20	19	12	
Niro EORTC PF	n=40	34	29	26	25	23	28	26	25	25	21	18	21	19	19	20	20	19	19	20	19	12	
Niro EORTC RF	n=40	34	29	26	25	23	28	26	25	25	21	18	21	19	19	20	20	19	19	20	19	12	
Pbo EORTC QoL	n=42	32	36	23	22	21	23	21	21	20	17	16	13	14	16	15	12	14	11	12	10	13	10
Pbo EORTC PF	n=43	33	36	24	23	21	23	21	23	21	17	17	14	15	16	16	14	14	12	13	11	14	10
Pbo EORTC RF	n=43	33	36	24	23	21	23	21	23	21	17	17	14	15	16	16	14	14	12	13	11	14	10

With S45F CTNNB1 Mutation



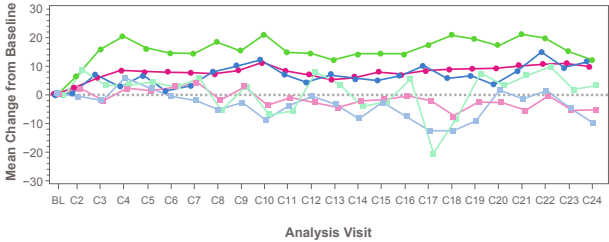
Niro EORTC QoL	n=13	12	10	6	5	5	8	8	8	8	5	8	5	6	6	6	7	7	6	8	7	7	6
Niro EORTC PF	n=13	12	10	7	5	5	8	8	8	8	6	8	5	6	6	6	7	7	6	8	7	7	6
Niro EORTC RF	n=13	12	10	7	5	5	8	8	8	8	6	8	5	6	6	6	7	7	6	8	7	7	6
Pbo EORTC QoL	n=16	10	12	7	11	8	7	8	9	6	8	6	5	5	4	5	5	4	4	4	6	3	3
Pbo EORTC PF	n=16	10	12	7	11	8	7	8	9	6	8	6	5	5	4	5	5	4	4	4	6	3	3
Pbo EORTC RF	n=16	10	12	7	11	8	7	8	9	6	8	6	5	5	4	5	5	4	4	4	6	3	3

With T41A CTNNB1 Mutation



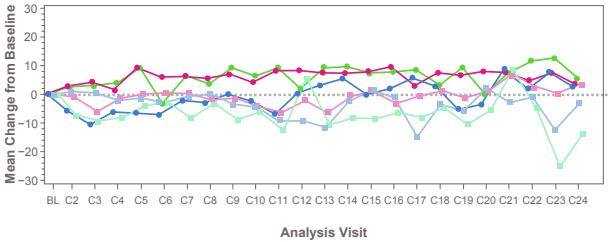
Niro EORTC QoL	n=24	20	21	18	16	13	17	14	13	11	9	9	11	10	9	8	9	8	7	8	10	9	7
Niro EORTC PF	n=24	20	21	18	16	13	17	14	13	11	9	9	11	10	9	8	9	8	7	8	10	9	7
Niro EORTC RF	n=24	20	21	18	16	13	17	14	13	11	9	9	11	10	9	8	9	8	7	8	10	9	7
Pbo EORTC QoL	n=21	18	18	12	12	11	7	11	9	8	5	5	5	6	4	6	6	4	4	4	2	2	
Pbo EORTC PF	n=21	18	18	12	12	11	7	12	9	8	5	5	5	6	4	6	6	4	4	4	2	2	
Pbo EORTC RF	n=21	18	18	12	12	11	7	12	9	8	5	5	5	6	4	6	6	4	4	4	2	2	

Presence of Pain at Baseline



Niro EORTC QoL	n=47	41	36	29	29	23	28	29	25	23	24	22	21	22	22	22	23	23	21	21	15		
Niro EORTC PF	n=47	41	36	29	30	23	29	28	25	23	24	22	21	22	22	22	23	23	21	21	15		
Niro EORTC RF	n=47	41	36	29	30	23	29	28	25	23	24	22	21	22	22	22	23	23	21	21	15		
Pbo EORTC QoL	n=45	34	40	24	25	23	24	19	16	17	18	15	14	13	14	12	8	12	9	10	12	9	5
Pbo EORTC PF	n=45	34	40	24	25	23	24	19	17	17	18	15	14	13	14	12	9	12	9	10	12	9	5
Pbo EORTC RF	n=45	34	40	24	25	23	24	19	17	17	18	15	14	13	14	12	9	12	9	10	12	9	5

Absence of Pain at Baseline



Niro EORTC QoL	n=23	19	17	16	13	15	14	14	15	13	11	9	11	12	9	13	9	11	7	9	10	8	6
Niro EORTC PF	n=23	19	17	16	13	15	14	14	15	13	11	9	11	12	9	13	10	11	7	9	10	8	6
Niro EORTC RF	n=23	19	17	16	13	15	14	14	15	13	11	9	11	12	9	13	10	11	7	9	10	8	6
Pbo EORTC QoL	n=24	17	19	11	15	12	10	16	10	8	8	7	7	8	7	9	7	7	5	3	6	4	5
Pbo EORTC PF	n=25	18	19	12	16	12	10	17	11	8	9	8	8	8	10	7	8	6	4	7	4	4	6
Pbo EORTC RF	n=25	18	19	12	16	12	10	17	11	8	9	8	8	8	10	7	8	6	4	7	4	4	6

The GODDESS DTIS physical functioning score is based on a 5-point Likert scale; a negative change from baseline value indicates improvement.
The EORTC QLQ-C30 is scored on a scale of 0–100; a positive change from baseline value indicates improvement.
BL, baseline; DTIS, Desmoid Tumor Impact Scale; EORTC QLQ-C30, European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire Core-30; GODDESS, GÖunder/Desmoid Tumor Research Foundation DEsmoid Symptom/Impact Scale; Niro, nirogacestat; Pbo, placebo; PF, physical functioning; QoL, quality of life; RF, role functioning.